Preclinical Model for Labeling, Transplant, and In Vivo Imaging of Differentiated Human Embryonic Stem Cells

Grant Award Details

Preclinical Model for Labeling, Transplant, and In Vivo Imaging of Differentiated Human Embryonic Stem Cells

Grant Type: Comprehensive Grant

Grant Number: RC1-00144

Investigator:

Name:	Alice Tarantal
Institution:	University of California, Davis
Type:	PI

Disease Focus: Kidney Disease

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $2,166,757

Status: Closed

Progress Reports

Reporting Period: Year 2

View Report

Reporting Period: Year 3

View Report

Reporting Period: Year 4

View Report

Reporting Period: NCE

View Report

Grant Application Details

Application Title: Preclinical Model for Labeling, Transplant, and In Vivo Imaging of Differentiated Human Embryonic Stem Cells

Public Abstract: The derivation and culture of human embryonic stem cells has provided new possibilities for treatment of a wide variety of human diseases because these cells have the potential to help regenerate and repair many types of damaged tissue. Diseases for which such cell-based treatments may be helpful include obstructive renal disease, a disorder for which there has been little progress made in terms of treatment. Infants with this and other inherited kidney disease may be severely compromised before birth and treatments necessary to prolong their life may be accompanied by severe side effects. This raises many difficulties not only for these young patients but also for their families. If new ways to treat these infants prior to birth can be developed, this could lead to the delivery of healthy babies at full term. The use of cells obtained from human embryonic stem cells to repair and treat damaged kidneys prior to birth offers promise to improve survival and quality of life for these babies. Since it is clear that embryonic stem cells have vast potential to form a variety of cell types, it is possible that the kinds of cells needed to provide repair could be obtained and treatments initiated prior to birth. The studies proposed will assess ways to obtain such cells and the effectiveness of such treatments. Ultimately, even small improvements in function of damaged kidneys following embryonic stem cell-based therapies may increase survival and eliminate the need for dialysis or kidney transplants. Although methods to grow embryonic stem cells and even obtain cells that could be useful for treating some human diseases have been described, the use of these cells for human therapies remains highly controversial because their safety remains untested. While these cells have great potential and promise to form cell types useful for treatment of disease, they also have the potential for uncontrolled growth and to form tissues that would be harmful. Therefore, studies must be performed and techniques must be developed to carefully examine the use of these cells in relevant models of human disease, and before they are ever considered for human treatments. The overall intent of these studies is to develop techniques that can be used to test the safety of human embryonic stem cell-based therapies, and to determine ways to evaluate the cells after they have been injected into the body. As we develop new treatments for obstructive kidney disease, we will use this model system to explore these essential safety questions related to stem cell therapies. The studies proposed will fill a critical need for new treatments for kidney disease, ways to monitor cells in patients, and develop methods to assess safety issues associated with the transfer of this research to human patients.

Statement of Benefit to California: This proposal focuses on ways to fill the significant gap in the development of new human therapies using stem cells – transfer of ideas and techniques that are developed in laboratories to effective and safe treatments for human patients afflicted with disease. While the potential medical benefits of human embryonic stem cells may seem great, proof that these cells will not cause harm must be shown, and this must be accomplished before any patients receive treatments. Removing the barrier preventing the transfer of promising stem cell therapies to human patients will require connecting people with the expertise to develop and to evaluate such treatments. With this in mind, our studies will bring together collaborators from many areas: developmental biologists, clinicians, engineers, and those with vast experience in the study of stem cells and with preclinical models to address questions associated with a pediatric kidney disease, which is one of the leading causes of chronic renal failure in children. Kidney disease is a major cause of illness and death among infants and children with over 20,000 babies born each year with kidney problems. Approximately 5,000 have kidney failure and are on dialysis or are in need of a kidney transplant. In California alone, nearly 100 children under 10 years of age are currently awaiting available kidneys for organ transplant. The benefit to the California community is, thus, potential new therapies for the treatment of kidney disease in children, and a model system available to all researchers in which safety and efficacy of embryonic stem cell therapies can be predicted.

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